

HEALEY ALS Platform Trial

Weekly Q&A – Oct 21, 2021



Healey Center

Sean M. Healey & AMG Center
for ALS at Mass General



The AMG Foundation

Guest Speaker

Xiaowei (Bill) Su, MD, PhD
Penn State Health, PA
Platform Trial Site Sub-Investigator



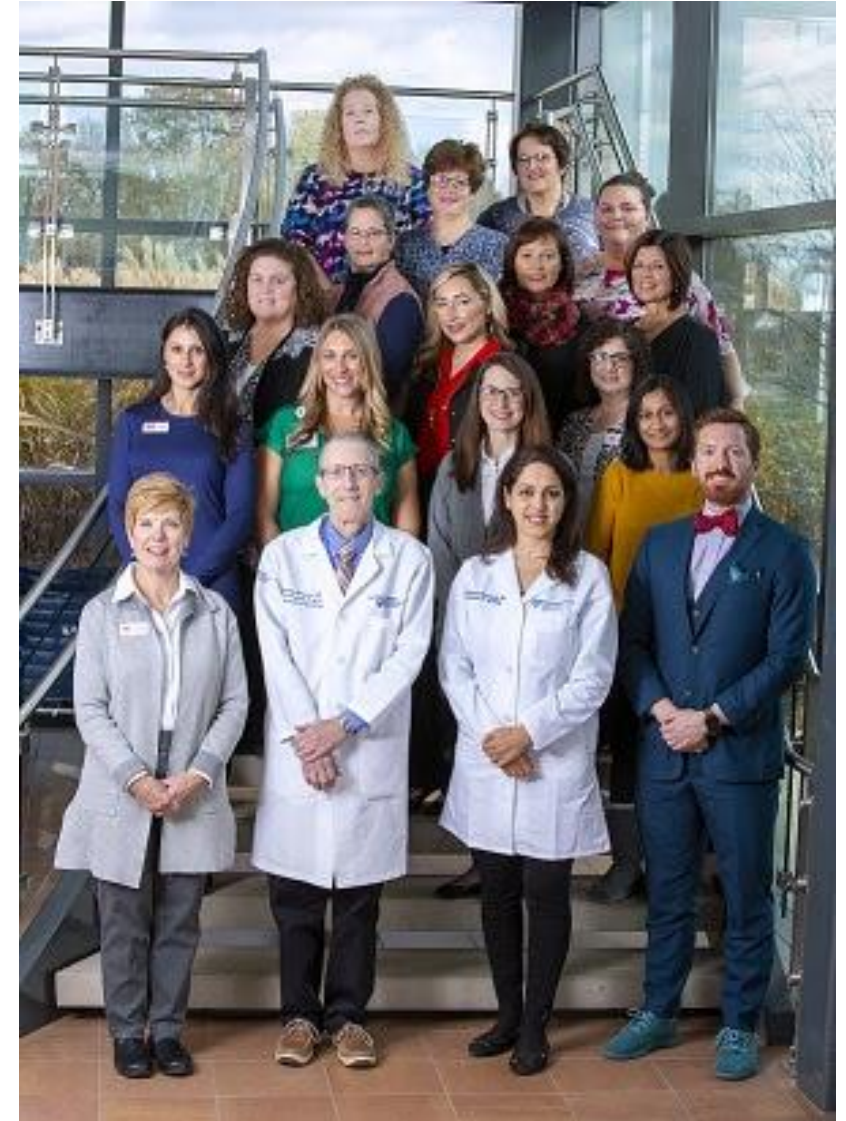
Penn State Hershey ALS Clinic Overview

Penn State Health M.S. Hershey ALS Clinic

- 220 active patients (80-100 new annually)
- 4 clinicians, 3 RNs

Penn State ALS Research Program

- One PI, 3 Sub-Is
- 3 Research Coordinators (2 Healey)



Penn State Healey Site Experiences

Healey has been exciting...

- Tremendous patient interest
- Highest enrollment in our ALS clinic & research program history

... And challenging

- Complex logistics & documentation compared to traditional clinical trials
- Ongoing assessment of how many research participants can be managed by each coordinator

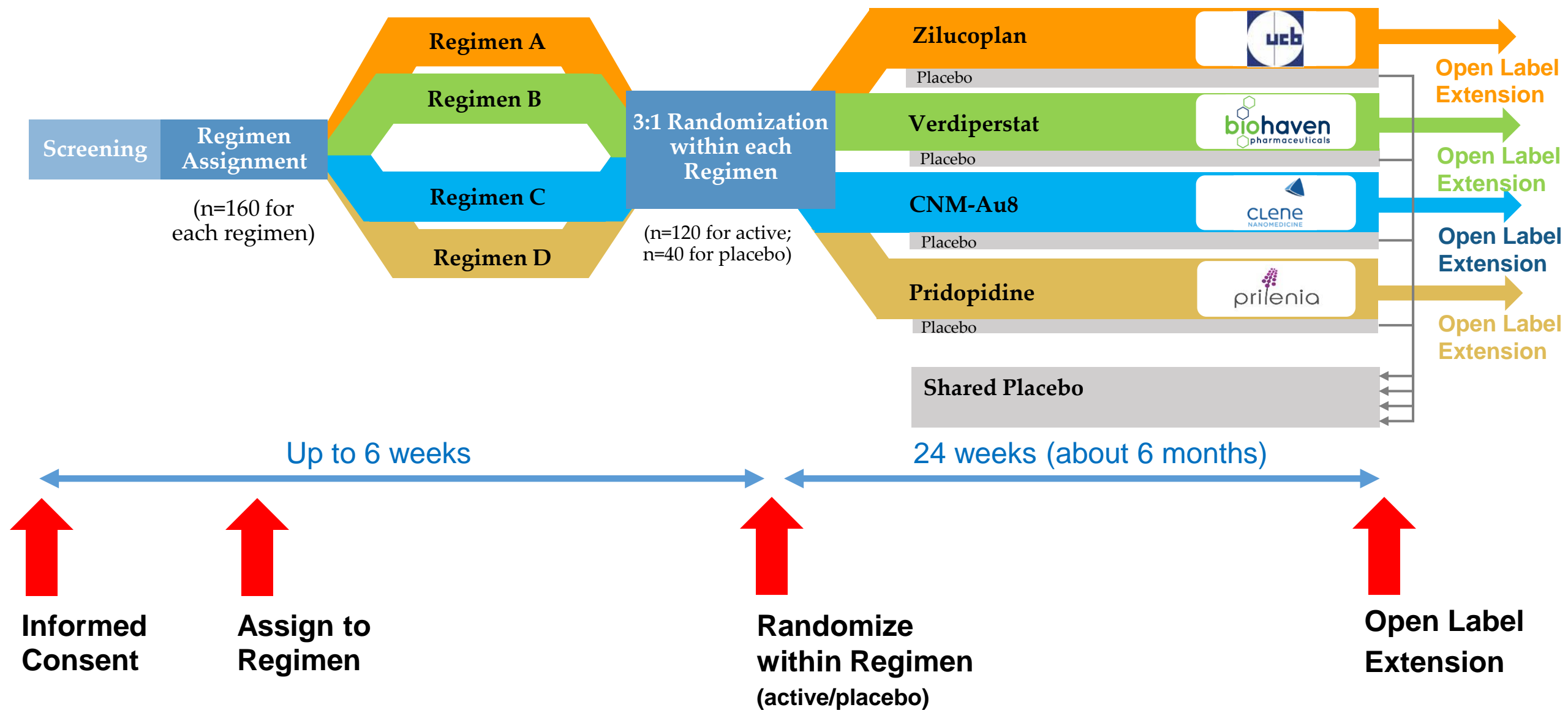
Support from MGH and central monitors greatly appreciated



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of October 21, 2021)

- **815** individuals with ALS signed informed consent
- **646** individuals were assigned to a regimen
- **584** individuals were randomized within a regimen (active or placebo)
- **239** have entered the Open Label Extension (OLE)

- **158** individuals were randomized within Regimen A
- **167** individuals were randomized within Regimen B
- **161** individuals were randomized within Regimen C
- **98** individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your partnership

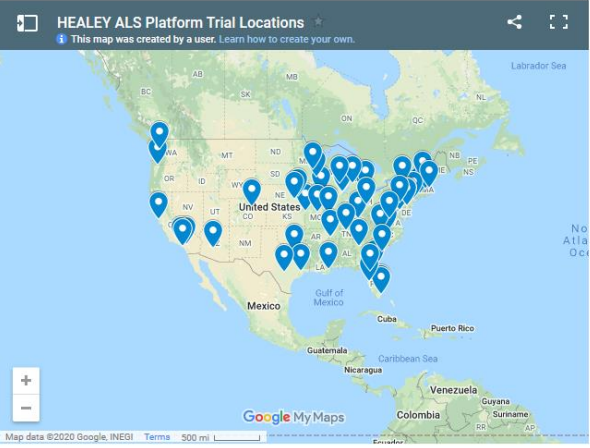
How to Find a Center Near You

<https://www.massgeneral.org/neurology/als/research/platform-trial-sites>

Sean M. Healey & AMG
Center for ALS

About the Healey Center Clinical Care Patient & Family Resources

Map of Participating Sites



HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

***New Site*-University of Southern California**

53 sites are actively enrolling



Plans for Expansion: 22 New Sites Coming Soon



- ✓ Atrium Health
- ✓ Cleveland Clinic
- ✓ George Washington University
- ✓ University of California, San Francisco
- ✓ Yale University
- ✓ Indiana University
- ✓ Stony Brook University
- ✓ Georgetown
- ✓ University of Arkansas
- ✓ University of Pittsburgh
- ✓ University of Southern California
- ✓ University of Utah
- ✓ Augusta University
- ✓ University of Cincinnati
- ✓ Virginia Commonwealth University
- ✓ Swedish Medical Center
- ✓ Las Vegas Clinic
- ✓ Kaiser, Los Angeles
- ✓ Lehigh Valley Health Network
- ✓ St. Alphonus Regional Medical Center
- ✓ Hackensack University
- ✓ Essentia Health

Note: Only those sites highlighted in blue are actively enrolling at this time. Additional sites will be activated on a rolling basis.

Patient Navigator



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To see whether you might qualify,
view the list of eligibility criteria:

<https://bit.ly/3p4PrLL>



Send us webinar ideas!

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- **Get to know our sites**



Upcoming Guest Speaker:

Oct 28th- Megan Miller, PhD (IAMALS Director of Science and Policy)